Chairman Michael C. Burgess, M.D.
Opening Statement
21st Century Cures Implementation:
Updates from FDA and NIH
July 25, 2018

(As prepared for delivery)

Today, we are here to conduct oversight and receive updates on implementation of one of the most substantial legislative accomplishments in the space of biomedical innovation – the 21st Century Cures Act. Cures passed both the House and the Senate with wide bipartisan support and was signed into law in December of 2016. As with all landmark laws, I think it is critical that the Congress, especially the relevant authorizing Committee, engage in oversight to ensure that the agencies are implementing the law successfully.

Based on the active leadership and robust activities of both the National Institutes of Health and the Food and Drug Administration, I look forward to hearing from Dr. Francis Collins, Director of the National Institutes of Health (NIH), and Dr. Scott Gottlieb, Commissioner of the Food and Drug Administration (FDA), regarding progress in implementing provisions of Cures implementation. I thank both of our witnesses for their willingness to testify on such an important topic.

The 21st Century Cures Act provides hope to those who need it the most – individuals and families suffering from life-altering, often life-threatening illnesses. Whether it be cancer, a rare disease, or Alzheimer's, there are countless conditions that are costly to Americans of all ages and their families. Sadly, we each know too well the financial and human toll that diseases place on our friends and communities.

One of the most impactful provisions in 21st Century Cures created the NIH Innovation Account in the Treasury. This account funds projects related to the Precision Medicine Initiative (PMI), the Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative, cancer research, and regenerative medicine. The pace and breadth of biomedical research continues to accelerate, as we now have treatments to cure diseases such as Hepatitis C, which was once unthinkable. Yet, there is still much we do not know, especially regarding neurodegenerative diseases.

21st Century Cures also included a provision to establish a National Neurological Conditions Surveillance System. Prior to Cures, there was no requirement or authorization to provide surveillance of neurological diseases. But this has changed thanks to the Cures law.

Specifically, this section of the law requires the Secretary of the Department of Health and Human Services to create such a system by expanding surveillance infrastructure and activities, including data collection to determine prevalence, risk factors, and diagnostic and progression markers. Preliminary results from an ongoing MS Society study show that there are nearly one million Americans living with MS, more than twice the previously reported number. The surveillance system included in Cures will provide us with better information so that we can further our understanding of, and eventually cure, these diseases. I am especially grateful to see progress on this important policy, as this part of the law began as a standalone bill that I introduced last Congress.

Additionally, Cures advanced precision medicine, which allows physicians to offer their patients truly personalized treatment. Achieving the full potential of precision medicine will require immense efforts to collect health care data in addition to research done by our nation's best research investigators. This law codifies the Precision Medicine Initiative and encourages the Secretary of HHS to carry out the goals of the initiative while ensuring confidentiality of patients' information. The *All of Us* Research Program is a major piece of the PMI and has already engaged over 1 million volunteers in the U.S.

Clinical trials play a crucial, and necessary, role in the drug approval process. While FDA's traditional clinical trial methods have proven successful, they are not always timely or applicable to new types of drugs. Cures requires the FDA to evaluate its trial designs and issue guidance for the purpose of "incorporating complex adaptive and other novel trial designs."

The innovation and promising results of efforts included in Cures will certainly provide Americans suffering from cancer and other diseases with the opportunity to undergo successful treatments, and in some cases, to be cured.

Thank you to Drs. Collins and Gottlieb for giving us updates on the implementation of this important law. I look forward to hearing your testimony.

I yield the balance of my time to the gentlelady from Tennessee, Ms. Blackburn, for a statement.